



RXi Pharmaceuticals

Investing for Cures 2017

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NASDAQ: RXII

Forward Looking Statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as “believes,” “anticipates,” “plans,” “expects,” “indicates,” “will,” “intends,” “potential,” “suggests” and similar expressions are intended to identify forward-looking statements. These statements are based on RXi Pharmaceuticals Corporation’s (the “Company”) current beliefs and expectations. Such statements include, but are not limited to, statements about the future development of the Company’s products (including timing of clinical trials and related matters associated therewith), the expected timing of certain developmental milestones, the reporting of unblinded data, potential partnership opportunities, the Company’s competition and market opportunity and pro forma estimates. The inclusion of forward-looking statements should not be regarded as a representation by the Company that any of its plans will be achieved. Actual results may differ from those set forth in this presentation due to risks and uncertainties in the Company’s business, including those identified under “Risk Factors” in the Company’s most recently filed Annual Report on Form 10-K and in other filings the Company periodically makes with the U.S. Securities and Exchange Commission. The Company does not undertake to update any of these forward-looking statements to reflect a change in its views or events or circumstances that occur after the date of this presentation.

Self-delivering RNAi (sd-rxRNA[®]) Platform

sd-rxRNA Competitive Advantages

Novel, self-delivering RNAi therapeutic compounds

Single compound incorporates gene silencing activity & cellular uptake

Robust uptake & silencing in multiple preclinical models

Demonstration of safety and clinical activity in human

Best RNAi technology for enhancement of cell-based therapeutics

Potential to expand CAR T-cell therapy to solid tumors

Provides for broad pipeline of RNAi drugs for unmet medical needs

RXi Pharmaceuticals' Development Pipeline

*In ACT

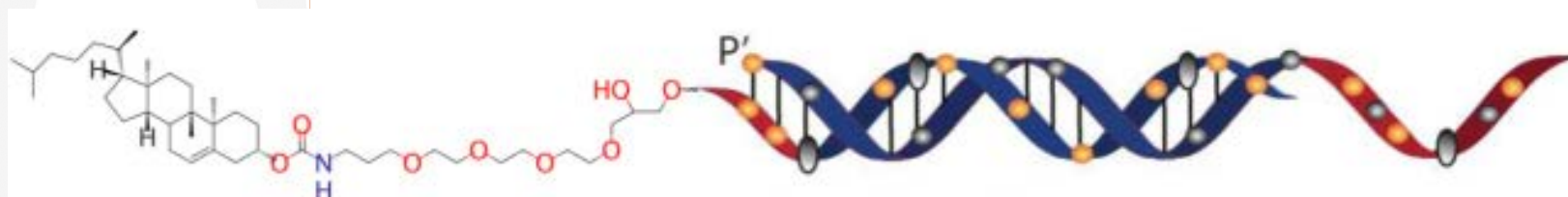
	Description	Indication	Discovery	Pre-Clinical	Phase 1	Phase 2	Phase 3
RXI-109	sd-rxRNA targeting CTGF	Dermal Scarring	[Progress Bar]		[Progress Bar]		
		Retinal Scarring	[Progress Bar]		[Progress Bar]		
		Corneal Scarring	[Progress Bar]				
RXI-762	sd-rxRNA targeting PD-1*	Immuno-oncology Solid tumors	[Progress Bar]				
RXI-804	sd-rxRNA targeting TIGIT*	Immuno-oncology Solid tumors	[Progress Bar]				
Undisclosed	sd-rxRNA targeting undisclosed targets	Immuno-oncology	[Progress Bar]				
Samcyprone™	Small molecule DPCP	Cutaneous Warts	[Progress Bar]		[Progress Bar]		

	Description	Application	Functional and Safety Testing	Consumer / User Testing
RXI-231	sd-rxRNA targeting tyrosinase	Uneven skin tone / pigmentation	[Progress Bar]	[Progress Bar]
RXI-185	sd-rxRNA targeting MMP1	Wrinkles / skin laxity	[Progress Bar]	



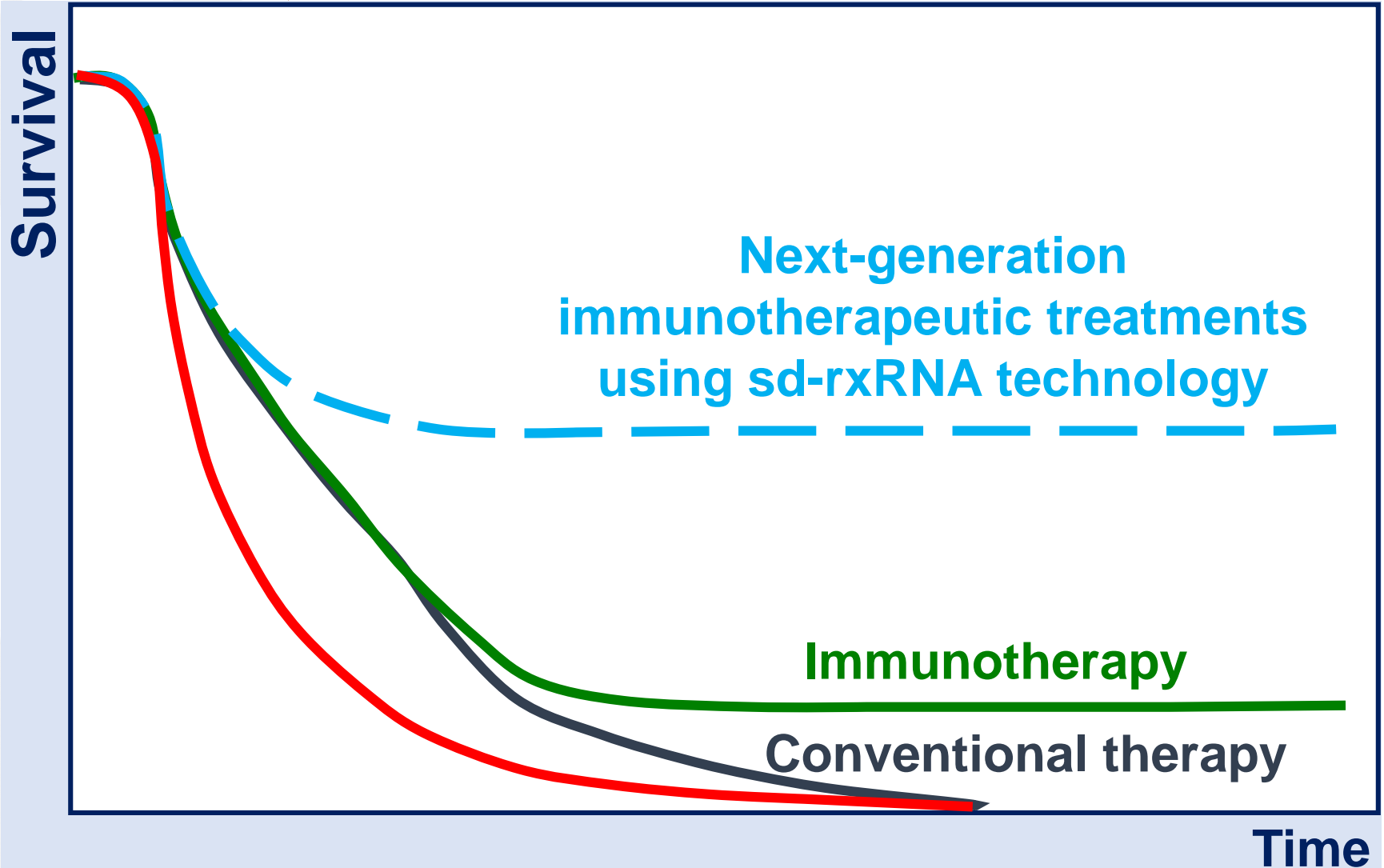
sd-rxRNA: Best RNAi Technology for Adoptive Cell Transfer

- One of the main challenges of RNAi technologies is the delivery of hydrophilic siRNA molecules to target cells
- Commonly used delivery techniques (lipid formulations, electroporation) are not optimal for *ex-vivo* treatment of therapeutic cells
- RXi's proprietary self-delivering RNAi technology provides simple and effective RNAi delivery for *ex-vivo* applications



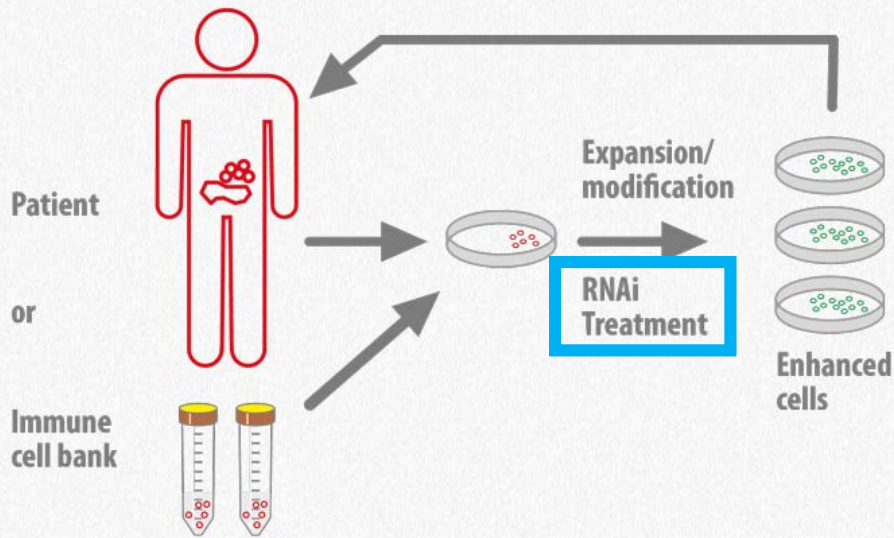
Promise to Cure Cancer

The Story of Immunotherapy



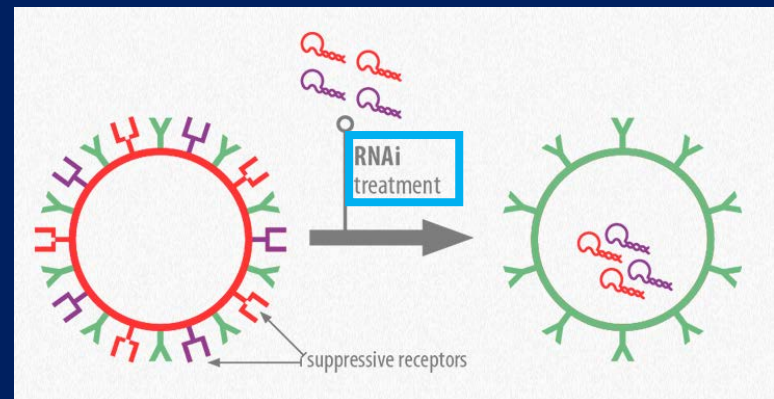
Overcoming Immunosuppression

Therapeutic Treatment with *RNAi*



Results in fewer suppressive receptors on the immune cell surface, which boosts their ability to detect and destroy tumor cells.

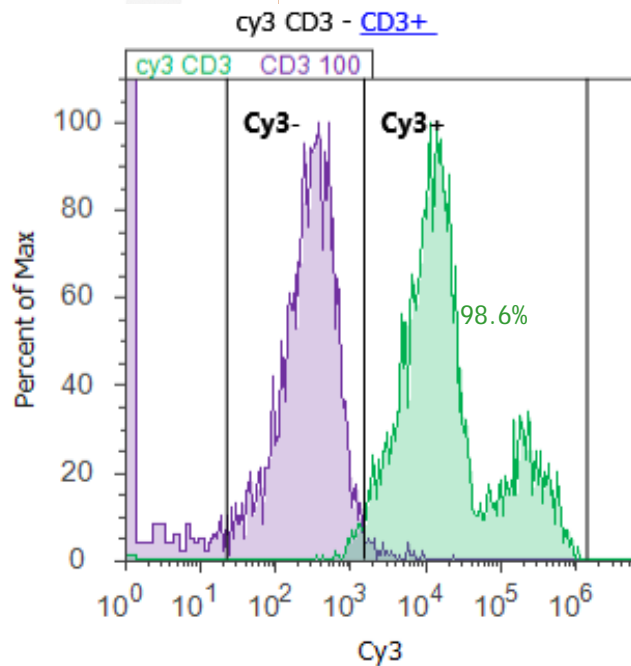
sd-rxRNA pre-treatment of cells can be used to silence one or more immuno-suppressive genes (such as PD-1 and other checkpoints).



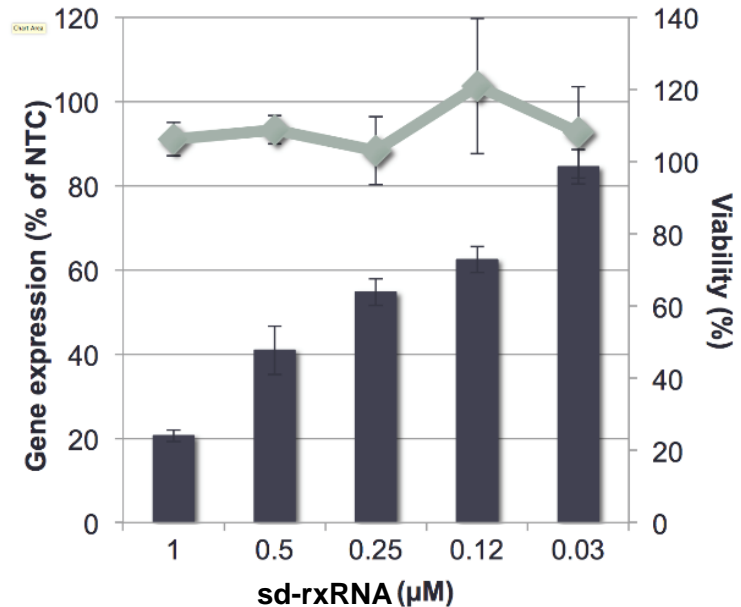
Immuno-oncology Program

sd-rxRNA Provides Highly Effective Transfection of Human T-Cells

Nearly 100% transfection efficiency...



... combined with high cell viability



sd-rxRNA is uniquely suited for checkpoint modulation in cellular immuno-oncology therapies

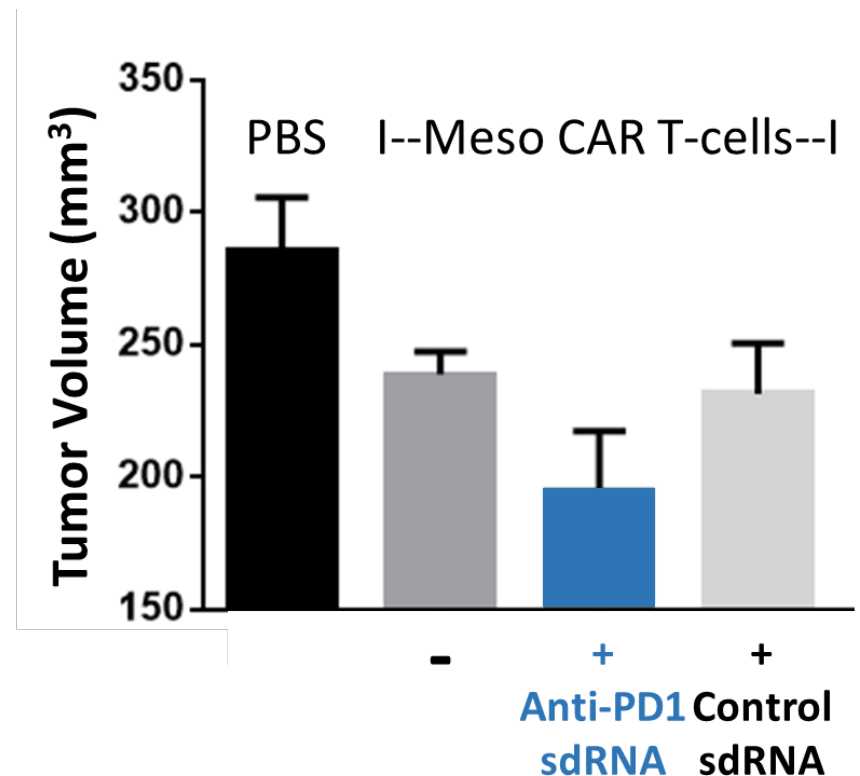
sd-rxRNA in Cell Therapy

Reduced *in vivo* Tumor Growth Using CAR T-cells Treated *ex vivo* with PD1 targeting sd-rxRNA

CAR T-cells Against Solid Tumors (at one month)

PD1 Silencing by sd-rxRNA Increases Long-Term Efficacy *in vivo*

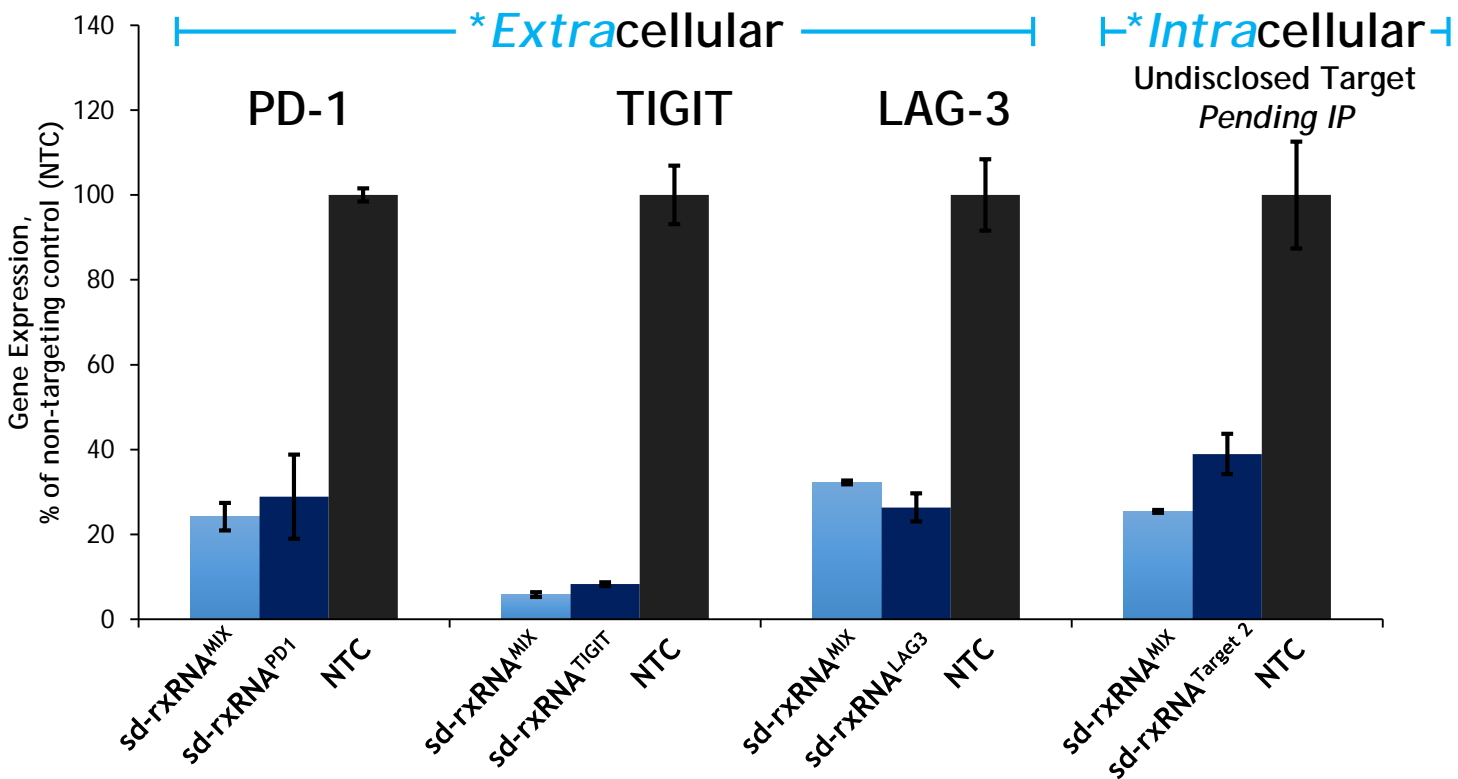
- Meso CAR T-cells: T-cells engineered to target mesothelin, overexpressed on many solid tumors
- Meso CAR T-cells were pre-treated with sd-rxRNA *ex vivo* and injected into human ovarian cancer tumors in mice
- Reduction of tumor growth is significantly improved by **anti-PD1 sd-rxRNA** treatment



¹CAR T-cells = chimeric antigen receptor T-cells

Targeting Multiple Immunosuppression Pathways* in a Single Therapeutic Entity

Simultaneous Silencing of Multiple Checkpoint Genes in T-cells is a Major Competitive Advantage of the Technology



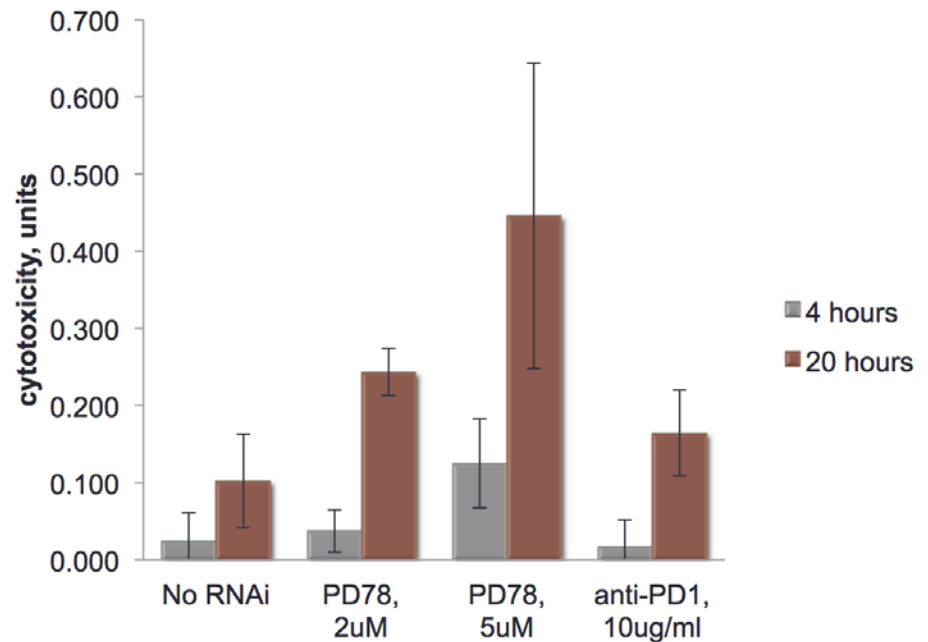
Next Steps: H2 2017 – Data on multiple checkpoint inhibiting sd-rxRNA compounds co-transfected in CAR T-cells in mouse models for solid tumors

sd-rxRNA in Cell Therapy

Tumor Infiltrating Lymphocytes (TILs) Against Melanoma

PD1 Silencing by sd-rxRNA in TILs Enhances Killing of Autologous Tumor Cells *In Vitro*

- TILs isolated from melanoma patient
- TILs treated with PD1 sd-rxRNA in a clinically used Rapid Expansion Protocol (REP)
- Tumor cell killing by TILs was measured by chromium release assay *in vitro*



Next Steps: H2 2017 – Publish *in vitro* results on use of sd-rxRNA with TILs in melanoma

sd-rxRNA: Safe and Versatile Approach to Reduction of Immunosuppression in Therapeutic Cells

	sd-rxRNA	Gene Editing	Antibodies
Off-tumor side effects	No	No	Yes
Persisting rogue cells in the body	No	Yes*	No
Cost of goods	Moderate	Moderate	High
Targeting multiple checkpoints	Easy	Difficult	Difficult

*Potentially further complicated by increased evidence of off-target mutations - Schaefer, K. A., Wu, W., Colgan, D.F., Tsang, S.H, Bassuk, A.G., & Mahajan, V.B. (2017). Unexpected mutations after CRISPR-Cas9 editing *in vivo*. *Nature Methods*, 14, 547-548. doi: 10.1038/nmeth.4293

Financial Overview

Cash and cash equivalents* (a/o 6/30/2017) ~\$7.7M

Burn rate ~\$2.5M/quarter

Cash runway Q3 2018
(Assuming current NASDAQ limitations)

Cash runway Q4 2019
(Assuming full use of \$15M equity line available)

Common shares outstanding ~24M
(a/o 8/2017)

Market Cap ~\$13.5M
(a/o 8/2017)

**Unaudited*

RXi Pharmaceuticals' Leadership

Board of Directors

Chairman of the Board

- Robert Bitterman

Directors

- Keith Brownlie
- Geert Cauwenbergh
- H. Paul Dorman
- Jonathan Freeman, PhD
- Curtis Lockshin, PhD

Management Team

President and CEO

- Geert Cauwenbergh, DrMedSc

Chief Development Officer

- Gerrit Dispersyn, DrMedSc

Chief Business Officer

- Alexey Eliseev, PhD

Vice President of Research

- Karen Bulock, PhD

Scientific Advisory Board

Ocular

- Peter Campochiaro, MD

Dermatology

- Jeannette Graf, MD
- Leroy Young, MD

Immuno-oncology

- James Griffin, MD
- Rolf Kiessling, MD

Oligonucleotide development

- Pamela Pavco, PhD